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(54) Title: MEANS AND METHODS FOR THE SPECIFIC MODULATION OF TARGET GENES IN THE CNS AND THE EYE AND METHODS FOR THEIR IDENTIFICATION

(57) Abstract: Provided are methods for the treatment of disorders of the central nervous system (CNS) and the eye. In particular, use of compositions comprising a compound capable of modulating a target gene or gene product is described for the preparation of a pharmaceutical composition for the treatment of disorders of the CNS and/or the eye, wherein the composition is designed to be administered outside the blood-CNS and the blood-retina barriers. Furthermore, methods are provided for identifying and obtaining nucleic acid molecules encoding polypeptides involved in CNS disorders or of the eye, methods for diagnosing said disorders as well as transgenic animal deficient in the expression of target genes identified in accordance with the described method. In addition, methods of identifying and isolating drugs that are particularly useful for the treatment of disorders related to the CNS and/or the eye are disclosed.

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